miRagen Therapeutics Presents New Clinical Data Suggesting Positive Impact From Systemically Administered MRG-106 on Mycosis Fungoides Form of Cutaneous T-Cell Lymphoma at EORTC Global Task Force Meeting

October 13, 2017

- *Improvements in skin disease observed in 22 out of 23 patients treated systemically through either subcutaneous or intravenous administration*
- *MRG-106 has been well-tolerated at all dose levels evaluated to date*

BOULDER, Colo., Oct. 13, 2017 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announced today new interim results from its ongoing Phase 1 clinical trial evaluating the safety, efficacy and pharmacokinetics of MRG-106 in patients with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). The data is being presented at the European Organisation for Research and Treatment of Cancer (EORTC) Cutaneous Lymphoma Task Force Meeting in London, United Kingdom, by Christiane Querfeld, M.D., Ph.D., Chief of the Division of Dermatology, and Director, Cutaneous Lymphoma Program at the City of Hope in Duarte, California.

“We are encouraged by the new MRG-106 Phase 1 data, as we observed that 96% of patients treated systemically experienced improvement in total skin disease at all dose levels evaluated,” said miRagen President and CEO William S. Marshall, Ph.D. “These findings suggest that MRG-106 has the potential to offer meaningful benefit to patients with CTCL. We are making what we believe to be important progress for patients in our MRG-106 Phase 1 clinical trial, and we were pleased to share these new data with the medical community.”

The MRG-106 Phase 1 trial consists of two parts. In the first part, patients were treated with 75 mg injections of MRG-106 directly into a specific lesion. These data, which showed a decrease in lesion size for all injected lesions, have been previously presented. The second part employed a multiple dose-escalation design to evaluate 300 mg, 600 mg or 900 mg subcutaneous or intravenous administrations of MRG-106. The newest results to be reported at EORTC include observations from four additional patients as well as longer term dosing data for existing patients who have continued on the trial. Interim data from the second part of the Phase 1 trial are summarized below:

- Twenty-two of twenty-three patients (96%) treated systemically showed improvement in total skin disease as measured by the maximal change in each patient’s modified Severity Weighted Assessment Tool (mSWAT) score, which assesses the severity of skin disease over a patient’s entire body.
    - Nine of thirteen patients treated for more than one month showed a 50% or greater maximal improvement in mSWAT scores.
    - Responses in mSWAT of 50% or greater appeared to be durable for all patients who continued on therapy. Additionally, to date five patients maintained a durable response for four months or longer.
    - The magnitude of mSWAT improvements appeared to correlate with amount of time the patient received MRG-106 treatment.
    - In patients who showed improvements, these were observed regardless of whether the patient was receiving background medication(s) for CTCL or MRG-106 alone.
- MRG-106 has been generally well-tolerated for all 27 patients evaluated to date at dose levels ranging from 75 mg to 900 mg.
    - Injection site reactions were seen in some patients after subcutaneous administration and were generally minor and self-limited.
    - Only one patient had a Grade 3 adverse event, worsening of pruritus (itchy skin) compared to baseline, which was considered potentially related to MRG-106 administration.

“The new MRG-106 Phase 1 data build upon those presented throughout the year, and to date has been generally well-tolerated with preliminary indications of clinical response,” said Paul Rubin, M.D., miRagen’s Executive Vice President of Research and Development. “We are encouraged by these results, which we believe support advancing MRG-106 into controlled clinical trials next year.”

The interim results announced by miRagen are from an ongoing Phase 1 trial in patients with documented mycosis fungoides. In part 1, patients were treated with 75 mg injections of MRG-106 directly into a specific lesion. Part two, of the trial which is currently enrolling, employs a multiple dose-escalation design to evaluate 300 mg, 600 mg or 900 mg subcutaneous or intravenous administrations of MRG-106. Patients were allowed to continue background medication provided these have remained unchanged for one month prior to receiving MRG-106. Enrolled patients receive a one week loading regimen followed by weekly dosing with 300 to 900 mgs through IV or subcutaneous administration. Patients are monitored for safety, and for improvement in skin disease via mSWAT at regular intervals throughout the trial.

MRG-106 is an inhibitor of microRNA-155. In CTCL, as well as certain other blood cancers, microRNA-155 is present at abnormally high levels, and may play a role in the proliferation of blood and lymph cells. miRagen believes therapeutic inhibition of microRNA-155 may reduce aberrant cell proliferation and the tumor growth characteristic of certain types of cancer.

miRagen plans to evaluate MRG-106 in additional oncology indications within the current Phase 1 trial, including adult T-cell leukemia/lymphoma, diffuse large B-cell lymphoma and chronic lymphocytic leukemia as in each case the disease process appears to be related to an increase in miR-155

levels.

Oral Presentation Details
Abstract title: Phase 1 trial evaluating MRG-106, a synthetic inhibitor of microRNA-155, in CTCL patients

- **Session:** Therapeutics (II)
- **Number:** 0-44
- **Date:** Saturday, October 14, 2017, 4:00 p.m. - 5:30 p.m. BST

For additional information, please visit the EORTC Cutaneous Lymphoma Task Force Meeting website: www.eortclondon2017.org

About miRagen Therapeutics, Inc.

miRagen Therapeutics, Inc. is a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in diseases where there is a high unmet medical need. miRagen's two lead product candidates, MRG-106 and MRG-201, are currently in clinical development. miRagen's clinical product candidate for the treatment of certain cancers, MRG-106, is an inhibitor of microRNA-155, which is found at abnormally high levels in malignant cells of several blood cancers, as well as certain cells involved in inflammation. miRagen's clinical product candidate for the treatment of pathological fibrosis, MRG-201, is a replacement for microRNA-29, which is found at abnormally low levels in a number of pathological fibrotic conditions, including cutaneous, cardiac, renal, hepatic, pulmonary and ocular fibrosis, as well as systemic sclerosis. miRagen is also developing MRG-110, an inhibitor of microRNA-92, under a license and collaboration agreement with Servier. MRG-110 is being developed for the treatment of heart failure and other ischemic disease. In addition to these programs, miRagen is developing a pipeline of preclinical product candidates. The goal of miRagen's translational medicine strategy is to progress rapidly to first-in-human studies once it has established the pharmacokinetics, pharmacodynamic, safety and manufacturability of the product candidate in preclinical studies. For more information, please visit www.miragen.com.

For information on clinical trials please visit www.clinicaltrials.gov.

Note Regarding Forward-Looking Statements

This press release may contain forward-looking statements that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements contained in this press release other than statements of historical fact, including statements regarding miRagen's strategy, future operations, future financial position, future revenue, projected expenses, prospects, plans and objectives of management or the expected features of or potential indications for miRagen's product candidates are forward-looking statements. The words "believe," "may," "will," "estimate," "continue," "anticipate," "intend," "plan," "expect," "predict," "potential," "opportunity," "goals," or "should," and similar expressions are intended to identify forward-looking statements. Such statements are based on management's current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation: that miRagen has incurred losses since its inception, and anticipates that it will continue to incur significant losses for the foreseeable future; future financing activities may cause miRagen to restrict its operations or require it to relinquish rights; miRagen may fail to demonstrate safety and efficacy of its product candidates; miRagen's product candidates are unproven and may never lead to marketable products; miRagen's product candidates are based on a relatively novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval, if at all; miRagen's product candidates may cause undesirable side effects or have other properties that could delay or prevent the regulatory approval; and the results of miRagen's clinical trials to date are not sufficient to show safety and efficacy of miRagen's product candidates and may not be indicative of future clinical trial results.

miRagen has based these forward-looking statements largely on its current expectations and projections about future events and trends. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described under the heading "Risk Factors" in miRagen's Annual Report on Form 10-K and subsequent periodic reports filed with the Securities and Exchange Commission. Moreover, miRagen operates in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for its management to predict all risks, nor can it assess the impact of all factors on its business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements it may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this press release may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. miRagen undertakes no obligation to revise or publicly release the results of any revision to such forward-looking statements, except as required by law. Given these risks and uncertainties, readers are cautioned not to place undue reliance on such forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement.

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Source: miRagen Therapeutics, Inc.